Intellia Therapeutics Highlights Strategic Priorities and Anticipated 2023 Key Milestones

January 5, 2023

- *Strategic priorities focus on late-stage development of its CRISPR-based medicines while continuing to expand and validate its industry-leading genome editing platform*
- *Submit IND application in mid-2023 as part of a global pivotal study of NTLA-2001 for the treatment of transthyretin (ATTR) amyloidosis with cardiomyopathy; study initiation anticipated by year-end 2023*
- *Submit IND application for NTLA-2002 for the treatment of hereditary angioedema (HAE) and initiate global Phase 2 study in 1H 2023*
- *Present additional clinical data in 2023 from both ongoing NTLA-2001 and NTLA-2002 first-in-human studies*
- *Progress first CRISPR-based gene insertion investigational therapy in humans; submit IND or equivalent filing for NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency (AATD) in 2H 2023*
- *Ended 2022 in a strong financial position with approximately $1.3 billion in cash*

CAMBRIDGE, Mass., Jan. 05, 2023 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage genome editing company focused on developing potentially curative therapies leveraging CRISPR-based technologies, today announced its strategic priorities for the upcoming two years as the Company enters its next phase of pipeline execution and platform innovation.

2023 – 2024 Strategic Priorities

1. **Initiate global pivotal trials** for Intellia's first two investigational *in vivo* CRISPR-based therapies, NTLA-2001 for transthyretin (ATTR) amyloidosis and NTLA-2002 for hereditary angioedema (HAE);
2. **Advance new platform capabilities to the clinic**, including CRISPR-based *in vivo* targeted gene insertion and a first-of-its-kind allogeneic cell engineering solution designed to avoid NK cell-mediated rejection; and
3. **Lead the development of new gene editing and delivery modalities**, compatible with its modular platform, which will extend Intellia's position of technological leadership and drive future pipeline growth.

"2022 proved to be another outstanding year for Intellia, with several significant clinical milestones achieved across our pipeline, further reinforcing the ability of our modular CRISPR genome editing platform to target a broad range of diseases," said Intellia President and Chief Executive Officer John Leonard, M.D. "These accomplishments reflect steady execution against our core strategy: to harness the immense power of genome editing, both for *in vivo* and *ex vivo* applications. As we look ahead, our highest priority will be to prepare for the initiation of global pivotal trials for our first two investigational *in vivo* CRISPR-based therapies, NTLA-2001 for ATTR amyloidosis and NTLA-2002 for hereditary angioedema. As these programs continue to progress, we believe we are moving closer to setting a new standard of care for people living with these and other serious diseases. In addition, we are advancing the next wave of platform capabilities, such as *in vivo* gene insertion and our proprietary allogeneic solution. Importantly, while the possibilities to apply our industry-leading genome editing technology are expansive, we are taking a disciplined approach with our portfolio by deploying resources on high-impact opportunities and collaborating with a network of other scientific leaders to expand the applications of our innovative technologies."

Based on these strategic priorities, which will be the Company's focus over the next two years, Intellia anticipates reaching the following key program milestones in 2023:

***In Vivo* Programs**

- **NTLA-2001 for ATTR amyloidosis**:
  - Submit an IND application in mid-2023 to enable inclusion of U.S. sites in a pivotal study of NTLA-2001 for patients with ATTR amyloidosis with cardiomyopathy (ATTR-CM).
  - Present additional clinical data from the ongoing Phase 1 study of NTLA-2001 in 2023.
  - Initiate a global pivotal NTLA-2001 trial for ATTR-CM by year-end 2023, subject to regulatory feedback.
  - Prepare for a Phase 3 study of NTLA-2001 for the treatment of ATTR amyloidosis with polyneuropathy (ATTRv-PN), including discussions with regulatory authorities.
- **NTLA-2002 for HAE**:
  - Initiate Phase 2 portion of the ongoing NTLA-2002 Phase 1/2 study in 1H 2023.
  - Submit an IND in 1H 2023 to support inclusion of U.S. sites in the Phase 2 study of NTLA-2002.
  - Present additional clinical data from the ongoing first-in-human study of NTLA-2002 in 2023.
- **Alpha-1 antitrypsin deficiency (AATD) franchise:**
  - Submit an IND or IND-equivalent application for NTLA-3001, Intellia's wholly owned insertion candidate in

development for AATD-associated lung disease, in 2H 2023.

- Complete IND-enabling activities for NTLA-2003, a wholly owned knockout candidate for AATD-associated liver disease, by year-end 2023.

- **Prevalent diseases:**
  - Progress one new *in vivo* development candidate, nominated in 2022, for the treatment of an undisclosed prevalent condition.

***Ex Vivo* Programs**

- **NTLA-6001 for CD30+ Lymphomas:**
  - Identify collaboration opportunities to advance development of NTLA-6001.
- **Additional *ex vivo* candidates:**
  - Advance multiple programs, wholly owned or in collaboration with partners, utilizing allogeneic platform.

Platform Innovation

- Advance novel gene editing technologies, including DNA writing, and delivery to other tissues outside of the liver.

Cash Position

- Intellia ended the fourth quarter of 2022 with approximately $1.3 billion in cash, cash equivalents and marketable securities.

About Intellia Therapeutics

Intellia Therapeutics, a leading clinical-stage genome editing company, is developing novel, potentially curative therapeutics leveraging CRISPR-based technologies. To fully realize the transformative potential of CRISPR-based technologies, Intellia is pursuing two primary approaches. The company's *in vivo* programs use intravenously administered CRISPR as the therapy, in which proprietary delivery technology enables highly precise editing of disease-causing genes directly within specific target tissues. Intellia's *ex vivo* programs use CRISPR to create the therapy by using engineered human cells to treat cancer and autoimmune diseases. Intellia's deep scientific, technical and clinical development experience, along with its robust intellectual property portfolio, have enabled the company to take a leadership role in harnessing the full potential of genome editing to create new classes of genetic medicine. Learn more at intelliatx.com. Follow us on Twitter @intelliatx.

Forward-Looking Statements

This press release contains "forward-looking statements" of Intellia Therapeutics, Inc. ("Intellia" or the "Company") within the meaning of the Private Securities Litigation Reform Act of 1995. These forward-looking statements include, but are not limited to, express or implied statements regarding Intellia's beliefs and expectations concerning: its ability to expand and validate its industry-leading genome editing platform, including the advancement of novel gene editing technologies, such as DNA writing, and delivery to other tissues outside of the liver; the safety, efficacy, success and advancement of its clinical programs for NTLA-2001 for the treatment of transthyretin ("ATTR") amyloidosis and NTLA-2002 for the treatment of hereditary angioedema ("HAE"), including the expected timing of data releases, regulatory filings, and the initiation, dosing, and completion of clinical trials, such as the submission of an investigational new drug ("IND") for NTLA-2001 in mid-2023 and for NTLA-2002 in 1H 2023, the initiation of a global pivotal NTLA-2001 trial for ATTR-CM in 2023, and the initiation of the Phase 2 portion of the ongoing NTLA-2002 Phase 1/2 study in 1H 2023; the advancement of development candidates, such as NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency ("AATD")-associated lung disease, NTLA-2003 for AATD-associated liver disease and NTLA-6001 for CD30+ lymphomas, including the timing and success of its IND-enabling activities and its IND or IND-equivalent submissions, such as the submission of an IND or IND-equivalent for NTLA-3001 in 2023; its ability to advance additional development candidates and timing expectations of advancing such development candidates and releasing data related to such technologies and development candidates, including candidates utilizing its allogeneic platform; its ability to optimize the impact of its collaborations on its development programs, including its collaboration with Regeneron Pharmaceuticals, Inc. and their co-development program for ATTR amyloidosis, and to advance additional development candidates; and its ability to identify new collaboration opportunities.

Any forward-looking statements in this press release are based on management's current expectations and beliefs of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to: risks related to Intellia's ability to protect and maintain its intellectual property position; risks related to Intellia's relationship with third parties, including its contract manufacturers, licensors and licensees; risks related to the ability of its licensors to protect and maintain their intellectual property position; uncertainties related to the authorization, initiation and conduct of preclinical and clinical studies and other development requirements for its product candidates, including uncertainties related to regulatory approvals to conduct clinical trials; risks related to the ability to develop and commercialize any one or more of Intellia's product candidates successfully; risks related to the results of preclinical studies or clinical studies not being predictive of future results in connection with future studies; the risk that clinical study results will not be positive; risks related to the development and advancement of novel platform capabilities, and risks related to Intellia's collaborations with Regeneron Pharmaceuticals, Inc. or its other collaborations not continuing or not being successful. For a discussion of these and other risks and uncertainties, and other important factors, any of which could cause Intellia's actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in Intellia's most recent quarterly report on Form 10-Q, as well as discussions of potential risks, uncertainties, and other important factors in Intellia's other filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Intellia undertakes no duty to update this information unless required by law.

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